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Non-primate EIAV-based lentiviral vectors as gene delivery system for motor neuron diseases.

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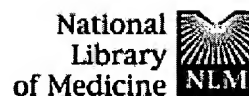
Motor neuron diseases such as amyotrophic lateral sclerosis (ALS) and spinal muscular atrophy (SMA) are neurodegenerative diseases, which cause progressive paralysis and premature death in affected adults and children. The treatment rational for these diseases is to halt or delay the degeneration of motor neurons. To date there are no effective drugs. This may however change with recent advances in gene therapy using lentiviral vectors. These vectors can transfer genes to motor neurons with high efficiency and give long term expression. One of the vector systems, based on the equine infectious anaemia virus (EIAV), can insert genes into the cells of the central nervous system after remote delivery including delivery into the muscle by exploiting retrograde transport pathways. This opens up the exciting possibility of rescuing the denervation of key muscle groups in patients by simple injections of these neurotropic lentiviral vectors into the muscle. This review will describe the general features of lentiviral vectors derived from EIAV. It will then describe some key examples of gene transfer and genetic correction in animal models of motor neuron disease. The prospects for the clinical evaluation of lentiviral vectors for the treatment of human motor neuron disease will be outlined.

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EIAV, CAEV and other lentivirus vector systems.

Olsen JC.

Cystic Fibrosis/Pulmonary Research and Treatment Center, University of North Carolina, Chapel Hill, North Carolina 27599, USA.

Lentiviruses that infect non-primates make up a diverse collection of viruses. Although these viruses have some features in common with HIV and other primate lentiviruses, differences in genome organization and viral gene function have made the successful derivation of vectors from non-primate lentiviruses unpredictable. Chapter discusses the construction and application of gene transfer systems derived from four non-primate lentiviruses including equine infectious anemia virus (EIAV), caprine arthritis encephalitis virus (CAEV), visna virus, and Jembrana disease virus (JDV).

Publication Types:

- Review
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